Participant Information Sheet - Interview

Research title

Survey and Interviews to elicit the Burden of Illness in Acute Hepatic Porphyria (AHP) Patient and Caregiver Members of the British Porphyria Association.

Invitation and brief summary

BresMed Health Solutions Ltd is an independent health economic and outcomes research consultancy contracted by the research sponsor, Alnylam Pharmaceuticals to conduct the study. The study will be conducted with the assistance of the British Porphyria Association. BresMed is conducting telephone interviews on the burden of acute porphyrias. We are inviting you to take part in this research. Participation is entirely voluntary.

What is the research for?

The purpose of the research is to better understand the impact of acute porphyria on you as a patient or as the caregiver of someone with acute porphyria.

What would taking part involve?

The research would involve a telephone interview which will take approximately one hour to complete. The telephone interview can be completed at any location with suitable privacy and telephone access and will take place between 4th February and 11th March 2019.

The questions are aimed to better understand the acute and chronic symptoms experienced, disease management, and impact on work, social life and overall quality of life.

Any adult with an acute porphyria or their main caregiver who complete the study survey may volunteer to also take part in an individual telephone interview. Approximately 15 patients and 8 caregivers will be interviewed from those who volunteer. Not all volunteers may be invited for interview. If more people volunteer than we have capacity to interview, volunteers will be selected to represent the broadest range of people living with acute porphyria.

Any information you provide will be treated as confidential. It will be combined with feedback from other patients or caregivers of those living with acute porphyria. You will remain anonymous unless you give permission to be identified. Even if you choose to give permission to be identified, no one involved in your care, including your GP or your porphyria specialist, would be informed that you have taken part in the research.

We are required to pass on to Alnylam Pharmaceuticals any details of side effects or product complaints relating to their products that are mentioned during the interview. This is to help them comply with applicable laws and regulations and learn more about the safety of their medicines. If this happens, we will need to collect details and report the side effects or product complaint. In this instance, you will be asked to give permission for us to pass your details to the company's drug safety department for follow-up. This will have no impact on the confidentiality and anonymity associated with the interview itself.

If during the interview it is evident a vulnerable individual is suffering or is likely to suffer significant harm, and a referral must be made to the appropriate authority without delay in order that the individual can be protected the interviewer must ensure that any disclosure of a confidential nature, which may be potentially harmful to the individual, must be dealt with in a sensitive and responsible manner.

To conduct the telephone interviews, we will collect and process your name, email address, telephone number or mobile number and an audio recording of the interview.

You will not receive any payment for taking part in this research.

What are the possible benefits of taking part?

You are not expected to benefit from the research, but the insights gained from this research could benefit future research and development in acute porphyria.

What are the possible disadvantages and risks of taking part?

You may consider the questions intrusive, however you do not have to answer any of the questions which you do not wish to answer.

The main burden associated with participation is the time taken to complete the interview, which is around one hour.

Further Information

What if something goes wrong? – If you have any complaint, in the first instance you can contact the study manager Nicola Mason nmason@bresmed.com If you feel your complaint has not been resolved to your complete satisfaction you can contact the study chief investigator Stephen Lombardelli slombardelli@alnylam.com The study chief investigator is an employee of the sponsor company Alnylam Pharmaceuticals.

What will happen if I don't want to carry on with the study? - You have the right to choose not to answer any of the questions or withdraw at any time without providing a reason for withdrawing.

How will my information be kept confidential? - Any information you provide will be treated as confidential. It will be combined with feedback from other patients or caregivers of those living with acute porphyria. You will remain anonymous unless you give permission to be identified. Your information will only be used for the research described and will not be passed to any other organisation without your permission. We will not keep any personal data you give us for longer than 12 months to allow completion of the combined data report. The research will comply with applicable data protection laws and the British Healthcare Business Intelligence Association legal and ethical guidelines.

For more information about your rights, or to obtain a copy of our privacy notice please contact Sarah Laycock by email info@bresmed.com You are also entitled to lodge a complaint with the supervisory authority, the Information Commissioner's Office (ICO), for the protection of personal data if you consider that your statutory rights have not been respected or that you have not received a response to your requests according to the law. The ICO is the UK's independent authority set up to uphold information rights in the public interest, promoting openness by public bodies and data privacy for individuals. You can contact the ICO at their helpline (0303 123 1113) or by visiting their website (https://ico.org.uk/make-a-complaint/).

What will happen to the results of this study? - The sponsor may use this anonymised information in three main ways: 1) to develop models of the personal and economic burden of acute porphyria, which support applications to groups which assess the value of new treatments. 2) as part of ongoing disease education to healthcare professionals and patient organisations. 3) in publications in medical journals or presentation at medical congresses.

Who is organizing and funding this study? - The research will be performed by BresMed Health Solutions Ltd - an independent health economic and outcomes research consultancy.

The research is being funded by Alnylam Pharmaceuticals – a pharmaceutical company. The chief investigator is an employee of Alnylam Pharmaceuticals.

How have patients and the public been involved in this study? - The British Porphyria Association have been involved in designing the survey and interview questions and may publish the anonymous results on their website and present results at patient meetings.

Who has reviewed this study? – This study has been reviewed by an independent research ethics committee.

For further information please contact Nicola Mason at BresMed Health Solutions Ltd by email nmason@bresmed.com